

Title (en)
PRECISE DELETION OF CHROMOSOMAL SEQUENCESIN

Title (de)
GENAUE DELETION VON CHROMOSOMALEN SEQUENZEN

Title (fr)
SUPPRESSION PRÉCISE DE SÉQUENCES CHROMOSOMIQUESIN

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Application
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Abstract (en)
[origin: WO2016179112A1] The present invention provides a method of treating a nucleotide repeat expansion disorder comprising delivering a pair of engineered nucleases, or genes encoding engineered nucleases, to the cells of a patient such that the two nucleases excise the nucleotide repeat responsible for the disease permanently from the genome. The invention provides a general method for treating nucleotide repeat expansion disorders and engineered nucleases suitable for practicing the method. The invention further provides vectors and techniques for delivering engineered nucleases to patient cells.

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